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Chair

Mr. Bill Casey

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● (0900)

[*English*]

The Chair (Mr. Bill Casey (Cumberland—Colchester, Lib.)): I will convene our meeting number 112.

Welcome to our guests. We appreciate your taking the time to share your wisdom with us. We look forward to hearing from you.

Today we have Catherine Parker, Director General, Biologics and Genetic Therapies Directorate, Health Products and Food Branch at the Department of Health. We have Karen Reynolds, Executive Director, Office of Pharmaceuticals Management Strategies; and Dr. John Patrick Stewart, Director General, Therapeutic Products Directorate.

I believe you've been here before, have you not, in this Parliament?

Dr. John Patrick Stewart (Director General, Therapeutic Products Directorate, Department of Health): I have previously.

The Chair: Welcome, and go ahead with your opening remarks.

Ms. Catherine Parker (Director General, Biologics and Genetic Therapies Directorate, Health Products and Food Branch, Department of Health): Good morning.

[*Translation*]

Thank you for the opportunity to be here today to discuss Health Canada's role in maximizing the quality, safety and effectiveness of drugs for rare diseases and in making these drugs available to Canadians.

Joining me today is Dr. John Patrick Stewart, and Ms. Karen Reynolds, also of Health Canada.

[*English*]

Rare diseases are life-threatening, debilitating or serious and chronic conditions affecting a small number of patients. There is no international standard for the definition of a rare disease. Many definitions include prevalence or incidence of the disease being targeted. Within Health Canada we have adopted a definition similar to that used within the European Union of a rare disease being one affecting fewer than five in 10,000 Canadians. Some of these diseases are considered ultra-rare and may even affect fewer than 10 Canadians.

However, although the number of Canadians with a particular rare disease may be low, there are thousands of rare diseases, and it is estimated that as many as one out of every 12 Canadians is affected by one.

Many of these diseases are genetically based and appear at birth or in early childhood. They can lead to a shortened lifespan and reduced quality of life and can place significant challenges on patients, caregivers and the health care system.

I understand you will hear directly from patients living with rare diseases. You will hear first-hand about the difficulties they often face in getting an accurate diagnosis, which can take years and require several specialists.

I now would like to explain the role that Health Canada plays in making drug treatments for rare diseases available to Canadians in need. We are aware of the challenges associated with developing and bringing to market drugs to treat rare diseases, which Canadians often refer to as orphan drugs. These challenges include limited to no information on the natural history of the disease and small patient populations, which make it challenging to conduct the typical clinical trials that are normally necessary to support drug development.

Health Canada oversees the testing of new drugs in clinical trials and their eventual authorization for sale, as well as the post-market surveillance. This includes drugs for rare diseases. Clinical trials represent a good opportunity for rare disease patients to access treatments and to contribute to supporting research to further understand their disease and the potential therapeutic benefit of new therapies.

Health Canada reviews clinical trial applications quickly and efficiently and provides free scientific advice to drug manufacturers on the design of clinical trials in small patient populations. Once a clinical trial is authorized by Health Canada, the trial is included in the clinical trials database. This database aims to help patients and their primary care providers to find available trials and it supports the recruitment of rare disease patients.

Once a drug manufacturer has sufficient evidence of a drug's safety and efficacy, it may seek a market authorization in Canada. Health Canada issues market authorizations for drugs, following an assessment of a complete dossier of information showing that a drug is safe, effective and of high quality, that the benefits will outweigh any risks, and that the risks can be managed. Once the drug is marketed, we continue to oversee it in order to monitor the safety profile.

Drugs that are intended to treat serious or life-threatening diseases, as many rare diseases are, are accelerated through the regulatory review process and given priority status or conditional approval. These accelerated pathways provide earlier access to promising new drugs for patients suffering from rare diseases.

However, a market authorization alone will not ensure availability. Canada's health care system is complex and involves the participation of multiple stakeholders who all have distinct roles in the planning and delivery of health care services. Health Canada's decision-making role in approving a drug is distinct from the roles of those who make decisions about cost-effectiveness, price setting and drug plan reimbursements.

Unfortunately, many rare disease drugs are very expensive. Health Canada, while recognizing and protecting its distinct role, must work with all of its partners and key stakeholders to improve access to drugs for rare diseases by encouraging the development and availability of safe and effective products.

● (0905)

Health Canada is receiving applications and approving drugs for rare diseases. Currently 30% to 40% of all new drugs approved in Canada, as well as in international markets, such as the U.S. and Europe, are drugs for these rare illnesses. Recent research from the Patented Medicine Prices Review Board indicates that nine out of the 10 top-selling orphan drugs are available in Canada. In 2017, 16 of the 36 brand new drugs we authorized in Canada are classified as orphan drugs in Europe or the United States. Most of these were reviewed and approved using our accelerated pathways.

Drugs that are not marketed in Canada may be accessed through Health Canada's special access program. This program provides access to unapproved medications on an exceptional case-by-case basis for practitioners treating Canadians with serious or life-threatening conditions when conventional treatments have failed or are unsuitable or unavailable. Approximately 30% of the drugs authorized through the special access program are used for the treatment of rare diseases.

However, we need to do more. Many jurisdictions have specific legislation in place to incentivize the development of rare disease drugs. The orphan legislation in the European Union and the U.S. were put in place to support the development of drugs that would otherwise not be profitable to bring to market.

In 2016, Health Canada launched the regulatory review of drugs and devices initiative, a major effort to improve the availability of and access to prescription drugs, including drugs for rare diseases. This represented a significant funding initiative in budget 2017.

We understand the particular needs and challenges of Canadians with rare diseases and have made a commitment to improve access to medications that treat these conditions. The regulatory review of drugs and devices initiative will make the regulatory process more efficient and better able to meet the needs of the health care system. It will also help to ensure that patients have access to those important new medications approved by Health Canada.

We will do this by working closely with the health technology assessment bodies to reduce the time between Health Canada approvals and reimbursement recommendations. We will also work with our health technology assessment bodies to provide parallel advice to industry on clinical trial designs at an early stage of drug development.

We will consider health care system needs in making decisions about which drugs Health Canada should prioritize. We'll use existing and new real world evidence to support regulatory decision-making across a drug's life cycle.

We will determine the best way to incorporate patient input into the regulatory approval process. Health Canada recognizes the value of information gathered from patients as the direct users of these products. This is especially true for rare diseases, given the limited information available and the fact that, for some of these diseases, patients and/or their caregivers may be the best experts.

Furthermore, Health Canada is renewing and modernizing the special access program to better meet the needs of physicians and patients. Improvements have already been implemented to provide greater assistance to physicians, and other changes are planned, including a new electronic system that will streamline requests.

In addition to helping to support interested parties navigate the regulatory framework, Canada's regulatory approach to drugs for rare diseases is now described online on Canada.ca.

We have heard that recognition of the orphan status of these products is important to many stakeholders. We now identify drugs that are considered orphan drugs in Health Canada's annual new drug authorizations report, which we have brought copies of for you.

We have sufficient flexibility under our existing regulations and policies to accommodate the challenges posed by drugs for rare diseases and make them available in Canada. Our ongoing regulatory review provides us with an opportunity to continue modernizing our regulatory approach to help support getting Canadian patients the medicines they need.

● (0910)

[*Translation*]

Now more than ever, it is a shared priority of Health Canada and its federal, provincial and territorial partners to improve the affordability, accessibility and appropriate use of prescription drugs in order to better meet the needs of Canadians.

[*English*]

The Chair: Thanks very much.

You have six seconds left to go. That's the way we like it.

We're going to go to questions, and I have one quick question.

How do you access the special access program? What door do you go to for that?

Ms. Catherine Parker: I will ask Dr. Stewart to answer that question.

Dr. John Patrick Stewart: The special access program is meant to be accessed by practitioners who have authority in provincial jurisdictions to prescribe unapproved medication. It's a process that begins with a physician making a decision that the unapproved therapy is the best choice for the patient in front of them given their condition and the current available therapies on the market.

Once they have made a decision that the best choice would be an unapproved therapy and that they are dealing with a serious and life-threatening condition, they complete a special access program request form, which is a two-page document that has five sections to complete. They submit that to Health Canada. It's reviewed by the special access program team to ensure that the request meets the requirement of the regulations, that there is actually a serious and life-threatening condition, that the therapies available on the market have been considered and tried or are unavailable, and that there is use on the safety and efficacy of that drug available to support the authorization.

The Chair: Thanks very much.

Now we will go to our panel, starting with Ms. Sidhu for seven minutes.

Ms. Sonia Sidhu (Brampton South, Lib.): Thank you, Chair.

Thank you, witnesses, for your testimony and presentation.

We know one in 12 Canadians is affected with rare diseases. Does the department look at the approval of rare disease drugs in other countries when approving those in Canada?

Ms. Catherine Parker: Yes, we certainly do. We're very much involved in collaboration with our international regulatory partners. At the time of a request for authorization, we consider whether a product has been approved in another jurisdiction, particularly with our partners in the United States Food and Drug Administration and the European Medicines Agency. We can get copies of any of their review reports and use them in our own decision-making.

We also participate in teleconferences with those two agencies almost on a monthly basis to discuss certain products that are in the approval process in all three, so that we share information during our review process as well.

● (0915)

Ms. Sonia Sidhu: The national program for newborn screening, with clear guidelines.... What do you think about DNA genomic screening? Do you think that's going to help, to screen?

Ms. Catherine Parker: That's a very difficult question to answer.

Dr. John Patrick Stewart: Certainly there's a trend in development of drugs to personalize medicine. Genomics and other markers can be used to hopefully identify subpopulations of patients with a disease where a therapy might be most beneficial, have the least number of side effects and cause the least number of problems.

This is a direction in drug development. Genomic-type testing is a big part of personalized medicines.

Ms. Sonia Sidhu: Thank you.

Some have suggested that pre-market clinical trials for drugs are not always reliable. Health risks are sometimes great.

In your experience with the challenges associated with pre-market trials for these drugs, how can the department address these challenges?

Anyone can answer.

Ms. Catherine Parker: I will comment and then pass it to Dr. Stewart.

We acknowledge that clinical trials have restrictions and may not reflect what happens once a drug goes into what we call real-world use. That is one of the reasons we have a conditional approval system for some of these products, where we can issue an approval of a medication on promising evidence from clinical trials, but with a commitment from the manufacturer that they are going to confirm that benefit and that safety profile once the product goes into use. That can be with confirmatory trials, registries, monitoring what happens in the actual usage and so on.

Dr. Stewart may want to add something to my answer.

Dr. John Patrick Stewart: I support what Cathy said. With orphan diseases—rare diseases—one of the challenges in studying drugs is the size of the population you're studying. Globally there may be fewer than 1,000 patients suffering from it.

Normally, in clinical trials that approve drugs for market authorization for sale, we're looking at phase three clinical trials with 3,000 to 5,000 patients, where statistics and the size of the studies allow us to have a better understanding of the performance and the risk associated. With rare diseases, we're talking about potentially 10, 20 or 30 Canadians with this condition.

The trials are often designed to be global, with many sites. There are challenges around who would be a controlled group and whether there is a controlled group. Often the participants are also the control group. We're faced with unique kinds of challenges around showing if there is effectiveness and safety with the drug. Globally we work on what is the evidentiary bar we would allow access to. As Cathy said, we also have to put in place conditions that allow us to continue to monitor the performance of the drug and whether the promising effectiveness that's shown in these early trials is borne out by more real-world use.

Ms. Sonia Sidhu: Thank you.

We know that this particular rare diseases group has a lot of challenges: misdiagnosis, unnecessary surgeries and financial hardship. For the orphan drugs, what were the key points of the framework? What work remains to be done to improve access to orphan drugs so more people can have access?

Ms. Catherine Parker: As the regulator, certainly, our priority is to make sure that as orphan drugs are being developed they come to Canada. We want to see sites in Canada for clinical trials so that patients can have access through that, and so practitioners can become familiar with the products. Also, when a company is ready to file for a market authorization, whether they're planning to file to the U.S. or the European Union, we want them file to Canada at the same time. That is the first step in access. It's to get those products through the approval system.

After that, of course, there are steps in the access chain, which we've described: the health technology assessment and also the reimbursement decisions. What we are doing within Health Canada through our regulatory review initiative is trying to bring some of those processes more into parallel rather than having them occur sequentially. Rather than an HTA proceeding after our approval, or mostly after our approval, we're trying to bring it into a parallel stream so that the regulator is working on the regulatory approval and the HTA body is working on the assessment for a funding decision at the same time, reducing some of those time lags between those. We feel that's a very important step in improving access.

Of course, there are other factors as well. Maybe my colleague Karen Reynolds could add to this.

● (0920)

Ms. Karen Reynolds (Executive Director, Office of Pharmaceuticals Management Strategies, Department of Health): Thanks very much for the question.

As Cathy was mentioning, ultimate access for many Canadians relies on a drug being reimbursed through either their public drug plan or a private plan. That's why the alignment with the health technology assessment is so important. As you're likely aware, agencies such as the Canadian Agency for Drugs and Technologies in Health and the Institut national d'excellence en santé et en services sociaux in Quebec are the ones that do these health technology assessments and make recommendations, largely to public plans at this time, in order for Canadians to have reimbursement.

The alignment that Cathy is talking about is really important, because at this time it could take several months before a drug is listed on a formulary, and patients will not have access until then, so we're trying to reduce that time and also align the evidence that both Health Canada and CADTH need, such that, again, when those processes are undertaken, they can happen faster.

The Chair: Thanks very much.

Ms. Gladu.

Ms. Marilyn Gladu (Sarnia—Lambton, CPC): Thank you, Chair, and thank you to the witnesses.

I want to preface my questions by saying that I do think that in Canada we have a good system in place that allows Canadians access to medications that many people in the world don't have access to, and I think we've created a climate where we're punching above our weight in clinical trials. What I'm going to focus on are areas where I have had situations that people in my constituency worry about.

First of all, with regard to the special access program, there were a couple of instances where individuals got hookworm when they went to the Caribbean and doctors prescribed things that were going to be available through the special access program. Both were denied. That had to be escalated to the health minister. Eventually they did get the drug, but I see that as one area where, when the doctor has prescribed it, I don't understand why people would not receive access. Hold that thought.

The second one is about people who have a lifelong condition and need a drug that's only available through special access. What's the one that...?

Mr. Ben Lobb (Huron—Bruce, CPC): It's Cystagon.

Ms. Marilyn Gladu: Cystagon is one of them. They get access to the drug, but then they have to reapply every few months for their whole life. It's a lifelong condition. It just seems that's a bit of bureaucracy.

The other thing I've heard is that there are drugs that have been on the list in the special access program for 27 years. It seems that at some point, if we're comfortable with the drug, it should somehow make its way onto the provincial formularies.

Will the special access program improvements you're talking about address those three scenarios?

Ms. Catherine Parker: I will refer that to Dr. Stewart to answer.

Dr. John Patrick Stewart: The short answer is yes. The special access program has been in operation for a long time and, as I mentioned earlier, it requires a dialogue with physicians, so the physician has to determine that specific therapy is the best choice for their patient.

They submit a request; it's assessed in the program and then it's ultimately authorized. There may, at times, be problems with documentation or lack of documentation, so there may be a further dialogue. If there isn't a response, it may ultimately be cancelled. If the dialogue with the physician is such that it is determined that it's not the best therapy, then it may be withdrawn.

Actually, formal denials are 0.3%. Last year there were 13,000 requests for the special access program, and only 48 were denied, so it's very rare.

Speaking to your question about process improvements, one of the things we've done is to look at when denials happen and ask how we can make that better. In the last year or two we've actually hired additional resources with clinical expertise, so we have a clinical pharmacist who actually works in the health care system as well as working for Health Canada. Before we make any denials, currently this individual, or someone with similar skills, will contact the physician and have a dialogue. Putting this in place has dropped our denials from about 1.5% to 0.3%, so there is a conversation.

You mentioned the situation with the drugs for the skin condition, larva migrans. We can't speak to specific requests, because that is private information, but I would just say that one of the bars that used to be met is that the request has to explain why the condition is serious and life threatening, and have the documentation on the use and safety and efficacy.

Sometimes requests will come in where that isn't clear. If you have certain conditions, like infectious diseases or the type of conditions that can be self-limiting or there can be aspects where it becomes life threatening, it requires a dialogue to determine whether the situation is serious and life threatening when it's approved.

Putting in place these additional clinical contacts I think will go a long way to avoiding situations where the problem is more clarity of information between the program and the practitioner.

As for other improvements, the communication is there. We've added clinical expertise. We've actually increased the collaboration across directorates and with the bureaus involved with approving drugs. You mentioned drugs that have been on the program for a long time, and this is an area where we are focusing and we've had dialogue with industry on why these drugs aren't coming in. Is it low volume or is it marketing decisions? We're putting in place a series of incentives to try to get companies that have had their drug on the program for so long to actually come in and market the drug.

We've had some success in the last year. We've had three of the top 10 drugs that were on the program for a number of years actually come in and get market approval, so that's a lot of help for pharmacists, for physicians and for hospitals, and we continue to work on that.

The other challenge you mentioned was patients who are on drugs for a long time on the program. Again, we've looked at this and we are changing some of our processes around situations where it's likely there will be a repeat request where the authorization period could be extended.

Having said that, the program is providing access to unapproved therapies that haven't gone through a formal assessment by the regulator to look at safety, quality, and efficacy. Some of these drugs are well known, and some are not, so the hesitancy to provide a long period of approval is that really they're not at the same bar. We don't have the same understanding as we do with approved drugs on the market in Canada.

● (0925)

Ms. Marilyn Gladu: Very good. Thank you.

My other question is for the PMPRB process.

I think the drug approval process we have today has gotten us to the median price of the OECD, which is why it was put in place, and we are doing lots of clinical trials.

Now, there are proposed changes. I sat with a bunch of stakeholders at the Macdonald-Laurier club. They were people from academia, people involved in clinical trials and people from pharma. Unanimously they were concerned that the changes to the process are going to make the process longer, that it will be almost three years before there will be any price certainty, and that this will cause people to not want to do clinical trials here, and actually have the unintended consequence of Canadians not having access to medications.

I heard that the process changes are now under re-evaluation. I just want a status update on that.

Ms. Catherine Parker: Karen Reynolds will answer that question.

Ms. Karen Reynolds: The changes you're referring to are proposed amendments to the patented medicines regulations, which would modernize the way the Patented Medicine Prices Review Board looks at setting non-excessive prices for patented medicine. They were published in the Canada Gazette last December. The consultation period on that closed in February. The department continues to evaluate the results of that consultation and continues dialogue with all the stakeholders before proceeding to Canada Gazette, Part II.

We don't have a definitive date for publication of the final regulations. We're certainly well aware of the comments and concerns of the stakeholder community. We can assure you that both the department and our colleagues at the board continue to work with those stakeholder groups, because we collectively understand the importance as a regulator of certainty, predictability and transparency in the application of those regulations to their ultimate success for Canadians overall and ensuring that Canadians continue to have access not only to the types of drugs that we're talking about today, but all patented medicines.

Thank you.

● (0930)

The Chair: Thanks very much.

Mr. Davies.

Mr. Don Davies (Vancouver Kingsway, NDP): Thank you, Mr. Chair.

Thank you to the witnesses for being here.

I want to read some excerpts from an article that was written in the National Post just last October, about 11 months ago. It's an article titled, "Health Canada gives 'kiss of death' to planned policy for rare-disease drugs". It says:

> Health Canada has quietly deleted from its website all references to a planned framework for rare-disease drugs that dates back to 2012 and was intended to improve the availability of such drugs in Canada. Canada is one of the only developed countries without a regulatory framework for rare-disease drugs, also known as orphan drugs.... Until Oct. 6, a Health Canada webpage claimed the department was "developing an orphan drug regulatory framework that seeks to encourage the development of orphan drugs and increase the availability of these products on the Canadian market." It also promised consultations that were "expected to take place before the end of 2017." The webpage has since been removed.

What happened?

Ms. Catherine Parker: You are correct that in 2012 the Minister of Health announced that Canada was going to proceed with the development of an orphan drug framework for Canada. We advanced a significant amount of work under that project. Most notable was the enactment of Bill C-17, Vanessa's law, which gave the regulator under the Food and Drugs Act many more powers that would be useful, especially in the rare disease space.

At that time we also considered going forward with a specific set of regulations. We decided on a change of course in this respect. We had conversations and dialogue with our international regulatory partners on some of the challenges they face because they had specific legislation for orphan drugs. We ultimately decided not to proceed with specific regulations, but through a regulatory review of drugs and devices initiative, we incorporated new processes and new pathways that could be very valuable for the approval of orphan drugs and also for some other products.

Mr. Don Davies: Did the consultations that were promised back in 2012 ever take place?

Ms. Catherine Parker: The consultations that were promised and that were on our website were consultations on upcoming regulations. We were no longer going to proceed with regulations, so we removed that reference to an upcoming consultation. We now have a full landing page on Canada.ca on our regulatory approach for orphan drugs.

Mr. Don Davies: One of the answers to that article on Health Canada was "many elements initially proposed as part of an orphan drug regulatory framework are now being considered more broadly for all drugs as part of this initiative". That's a direct quote from Health Canada.

Ms. Catherine Parker: Yes.

Mr. Don Davies: I have two questions.

One, have you taken a focus that was originally on rare disease and orphan drugs and now subsumed that in a larger review of all drugs, as the quote suggests?

Two, when do you expect that review to be complete?

Ms. Catherine Parker: Yes, I will add caution by saying that we are seeing that many more of the applications that come to Health Canada are for orphan indications.

Relating to the question on genetic testing, as Dr. Stewart said, we're seeing a lot of medications coming through where as a result of genetic work, they are able to identify a subset of a disease. The disease itself may not be orphaned, but the subset is orphaned. We're seeing much more of that, so when we talk about what we're doing for all drugs, we are seeing a large proportion of orphan drugs.

One of the concerns we had going forward with specific regulations was that you have to tie them to a specific definition. Most definitions are based on incidents. For example—

Mr. Don Davies: Excuse me, but you're straying very far away from my questions and I have limited time.

Ms. Catherine Parker: Okay.

Mr. Don Davies: When is the review expected to be complete was my question.

Ms. Catherine Parker: We have already launched a number of achievable items in the regulatory review of drugs and devices. We are planning to have the entire initiative completed by the end of 2021.

However, we have already launched a number of things. We now offer aligned reviews between the regulator and the HTA to all drug manufacturers. We are providing early advice in drug development to sponsors in parallel with the HTAs. We are developing specific regulations that will allow us, for certain products, which has a great applicability to orphan drugs, to recognize a foreign regulator's decision to approve.

Those are—

● (0935)

Mr. Don Davies: Thank you. I appreciate that.

You mentioned that the EU has legislation. In the article, it says that Canada is a country that doesn't have any regulatory regime.

I take it that's correct.

Ms. Catherine Parker: We don't have any specific regulations.

Mr. Don Davies: Okay.

You said that the EU has legislation. What would be the key parts of the regulatory system or legislation that other jurisdictions like the EU have? Are they making a difference? Are they helpful?

Ms. Catherine Parker: In countries that have legislation, they have a number of things to encourage development. Some provide tax incentives for development. Some provide extended market exclusivity periods. There are also provisions for medications to be eligible for accelerated review, which we already have in Canada.

Those countries that have legislation have a number of components in that legislation. Our priority in Canada is to get the drugs here at the same time as they come to the U.S. or Europe. We are more focused on getting them into Canada and getting them through the regulatory process in the quickest and most efficient way, and working to also get access to them.

Mr. Don Davies: Thank you.

The Chair: We're going to Mr. Ayoub.

Mr. Ayoub I suspect will be asking his questions in French.

[*Translation*]

Mr. Ramez Ayoub (Thérèse-De Blainville, Lib.): Thank you, Mr. Chair.

I would like to thank the witnesses for being with us.

There is another extremely interesting and in many cases very urgent subject, since we are talking about rare diseases and access to drugs for a small part of the population.

I would like to know what Health Canada's mission is in relation to the approval and approach for rare diseases.

What is your mission with regard to this approach?

Ms. Catherine Parker: Thank you for your question.

[*English*]

Our mission as Health Canada is to provide availability of safe, effective and high-quality medications. That is applicable both to the rare disease community as well as the disease community at large.

[*Translation*]

Mr. Ramez Ayoub: I imagine that by setting up this mission, you had developed a plan to make it a success. How satisfied are you at the national level? Indeed, there are large gaps in Canada in access to drugs.

How does Canada compare on the international scene with our neighbour country, which we often criticize?

According to the Canadian Organization for Rare Disorders, 60% of drugs from the United States are accepted in Canada and when they are, it is after six years.

When you say that you want to have the drugs along with the other countries, whether it's Europe or the United States, that doesn't seem to be the case.

What is our rank and where are we going with all this? What is your action plan for the coming months?

Ms. Catherine Parker: Thank you for your question.

[*English*]

It is correct that we are not seeing that every medication which goes to the U.S. or Europe is coming to Canada at the same time.

There are two issues: first of all, getting the medication here, and also getting it at the same time as those other countries. We are seeing some significant improvements in that. There used to be long lag times between filing to the U.S. and to Europe and then Canada as the third country of filing, but it would sometimes be a very long lag period. We are seeing significant improvements in that, and we're seeing that many submission filings are concurrent or overlapping.

That is our first priority, that—

[*Translation*]

Mr. Ramez Ayoub: What is the reason for this improvement? What is it that makes some cases improve, while others do not? What makes success not fully offset failure? Why doesn't this work for all drugs? Why are we not at the same level as all other developed countries?

[*English*]

Ms. Catherine Parker: We are in dialogue with the pharmaceutical industry as to why there is still a difference in times of filing. They give us a number of reasons.

They find it difficult sometimes to work with more than one regulator at the same time, so we are trying through international co-operation and work sharing to minimize that obstacle. We are actually encouraging through a number of our projects that companies can file to Canada and another regulator at the same time, and we will work share on those submissions so that they are basically only dealing with one party.

There are other factors, of course. Unfortunately, it is a business decision for manufacturers, the whole drug marketing, so we are doing as much as we can to encourage that information.

We are internationally harmonized with respect to the requirements for drug approval so that companies can file one dossier that is applicable to all regulators. Also, we have what we call a common portal with the U.S. FDA so companies can now file simultaneously for approval to Canada and the U.S. through one common filing portal. That has improved the situation.

● (0940)

[*Translation*]

Mr. Ramez Ayoub: I accept your answer, but it doesn't reassure me about short-term improvement.

I put myself in the shoes of Canadians with a rare disease who tell their MP and government that they can't get treatment in Canada, that they must go to the United States or Europe. They must seek hope of being treated outside of Canada. But we can't accept this. If Canadians are going to be treated outside Canada, it is because we haven't been able to provide them with the service they need.

I understand that an effort is being made to reduce the gaps, but what is your plan to completely eliminate this gap, despite the economic pitfalls and marketing difficulties of pharmaceutical companies? Canada must become a leader and be among the first in the world.

Ms. Catherine Parker: Yes.

Mr. Ramez Ayoub: Having said that, I don't have a plan, I haven't found one, but you aren't reassuring me, at least not today. I would like you to reassure me. You have one minute left to do so.

[*English*]

Ms. Catherine Parker: Did you want to add...?

Mr. Ramez Ayoub: Mr. Stewart.

[*Translation*]

Dr. John Patrick Stewart: I have one thing to add. Our regulations do not give

[*English*]

authority to compel a sponsor to come to Canada. We cannot compel a company to market a product in Canada, so we work to encourage them to come. We work to provide incentives at the level of clinical trials. We work to ensure that Canadians get access if there are rare disease trials going on. Canada punches, I think, above its weight from the point of view of academia and research centres, so we try to get these trials in so Canadians get access at the development stage and therefore we have evidence on Canadian patients when the submission comes in.

At the end of the day, we cannot make a company come to Canada. One of the realities is the population in Canada is not the same as the population of the U.S. or in the EU, so some of the decisions of these larger companies when they come in in their sequencing is the size of the population.

As Cathy pointed out, we are trying to work internationally, work sharing, so that companies can come in and get an approval for more than one country with one application. We're hoping this will be an incentive for companies to look to Canada and our partners we're work sharing with to come sooner.

[*Translation*]

Mr. Ramez Ayoub: Thank you.

[*English*]

The Chair: That completes our questioning for this panel.

You've completed your chore here today and we thank you very much. We only scratched the surface of this very complicated issue, but we do appreciate your sharing your information with us and your expertise.

Ms. Catherine Parker: Thank you very much.

The Chair: Thank you very much.

We're going to suspend for a minute while we change panels.

● _____ (Pause) _____ ●

● (0945)

The Chair: We will now begin with our second panel.

Today we have Tammy Moore, chief executive officer from the ALS Society of Canada. From the Canadian Organization for Rare Disorders, we have Maureen Smith, Board Secretary, and we welcome back Durhane Wong-Rieger, President and Chief Executive Officer.

Welcome. We're looking forward to your testimony.

I'm not sure who's going to start. Do you have an order?

Dr. Durhane Wong-Rieger (President and Chief Executive Officer, Canadian Organization for Rare Disorders): We'll go first.

The Chair: Perfect.

Dr. Durhane Wong-Rieger: First of all, a huge thanks to the committee for having this very special panel and certainly, for inviting us again. We are really pleased that you're undertaking, for the first time, a focused look at Canadians with rare disorders. This is a huge asset to us.

I especially want to thank Ben Lobb—who I hope is somewhere around here—and also ex officio member John Oliver for their leadership within this caucus to make this study happen.

To those of you who are health professionals here, and I know some of you are, we also would like to build on what you've already heard from your constituencies and your patients as well around the first-hand impact of rare diseases.

Certainly, we're very pleased with the way you've asked the question. What are the challenges and barriers, but also, what are some of the solutions? I think that's what we all really want to get to.

We very much appreciated hearing the Health Canada presentation as well. We'll add a little bit to what you've heard and to some of the very cogent questions that you folks raised with that.

This is, in fact, as you have seriously addressed here, a long-neglected part of Canada's health care system. I think, as you've already heard, and I don't need to reiterate, rare diseases affect nearly three million Canadians directly, and that's not including family members. Two-thirds of those are children. Most of these diseases are disabling, and that's why they're so important. They're lifelong conditions, and many of them are life-threatening unless we diagnose them and can intervene quickly.

The good news, of course, is that we are doing a much better job in terms of diagnosis. There are, in fact, many more treatments coming. We just did another survey this past June, and we will be able to break down the results for you to look at, but about 80% of people with rare diseases who responded to our survey were clear. They have challenges getting access to the medicines they need. I think Maureen, who appeared with me last time, is going to be talking with you about her challenges of getting diagnosed and treated for her rare condition.

At CORD, we've been working for many years to address these challenges. One of the things that we did in the context of this was the launch in 2015 of Canada's rare disease strategy, which we were very happy to do here at Parliament in order to have the recognition of the members of Parliament in support of that launch.

One of the key goals—I'm not expecting you to remember, but I will reiterate for you—is, in fact, access to promising therapies. We're very pleased to be here today to talk to you about some of the challenges that patients face in accessing those therapies and about some of our recommendations, especially how the federal government can help improve access.

Before I get started, I will turn it over to Maureen Smith. Not only is Maureen a patient, but she has also been for many years the secretary of the board for CORD. She's going to share her story.

● (0950)

[*Translation*]

Mrs. Maureen Smith (Board Secretary, Canadian Organization for Rare Disorders): Mr. Chair, committee members, thank you for inviting me to appear before you today.

As Ms. Wong-Rieger said, my name is Maureen Smith, and I am a Canadian with a rare disease. It was diagnosed when I was eight years old, after four years of tests and visits to specialists.

For the past five decades, access to treatment and drugs has played a key role in my life.

Over the past 10 years, as a volunteer with the Canadian Organization for Rare Disorders and many other groups and advisory committees, I have heard from many Canadians with rare diseases. I have also been able to learn about the difficulties faced by other stakeholders.

I am delighted to share with you my perspective as a patient.

[*English*]

I thought long and hard about having this opportunity and giving you one message, if I could. I'm going to give that to you.

When you have a rare disease, you face medical uncertainty, and often there's nothing that can be done about that. In my own case, I was on the first Canadian clinical trial—four children with an ultra-rare disease. I was the only girl. You live with that medical uncertainty your entire life. Often, even with the best interest, there's nothing that can be done about that.

When you add the burden of uncertainty of access to treatments, that just adds another layer to your life. If you're fortunate enough to have a treatment, then you constantly worry about whether you're going to be able to access that treatment. If you do get it, then you worry about it being taken away.

I wanted to share that with you. That is one of the most difficult things for patients to deal with. A lot of medical uncertainty can't be dealt with, but I'm hoping that you will try to lessen that burden on Canadians who have rare diseases, and their families.

The Chair: I just want to caution you that in your opening remarks you were quite long. You only have about five minutes left to go.

Dr. Durhane Wong-Rieger: Let me pass over some of it. From listening to your questioning before, I understand that this committee is actually quite cogent with some of the initial challenges, so I won't go through all of them with you. I will highlight a few.

We do endorse what Health Canada is doing right now in terms of an orphan drug regulatory approach. They brought it together, but we do reiterate that having an orphan drug regulatory framework would actually bring some additional supports and advantages for bringing those therapies into Canada. That is a very important consideration still, but as I say, we do endorse what they're doing now.

The big challenge, as I think you have heard from Cathy as well, is that having them approved does not mean access, and a lot of the access is in fact in the reimbursement system. Certainly, we think that aligning the review process between Health Canada now and the health technology assessment agencies could reduce the timing. Quite frankly, what we are very concerned about is the health technology assessment process that we use in Canada by CADTH and by INESSS is not appropriate for rare diseases.

What we do know is that the majority of those drugs which go through that process are in fact.... Originally, they were being denied. Now they're denied because they're not deemed cost-effective. They do not meet the standards of cost-effectiveness that have been set for more common drugs. I think you have already indicated that you can understand why there are so many challenges in terms of drug pricing, the uncertainty, and the long-term benefits of those drugs. Those are the two parameters that have CADTH and INESSS coming back with not only negative recommendations but also recommendations of a discount, sometimes as much as 97% of the price, in order for them to be approved.

It isn't a matter that they've taken a few more months in getting there, as Dr. Stewart says. It's a matter of two years. It's a matter of four years for them to negotiate that price. That's a tragedy. That's a time when, for patients with life-threatening, debilitating disorders, either their diseases get worse or, in many cases, they get to the point where the drug is no longer working for them because they've progressed too far, or they die. This is not good.

We've asked patients what's worse, not having any drug, or having a drug that you can't get access to. You can imagine what the answer is. Sometimes it's worse to not be able to get access to a drug that could in fact be beneficial to you, and that's the case.

We do want to make sure that there is more put into the assessment of these drugs, but we need a separate HTA pathway, like the U.K., France and what Australia has just come out with, and to recognize that we need to do that differently. Again, we think the federal government can help support that tremendously.

We do want to highlight the fact that, as you've heard, the PMPRB price and regulations are in fact.... We have no doubt about it, because companies have talked to us about it. It will have a devastating effect in terms of bringing in those drugs in a timely way, as we've talked about. We will not have them available to us. We will not be a first-tier country anymore. We would definitely not get the clinical trials in, because no company is going to come to Canada where the pricing is set in such a way that it's different from every other country, and where they are not going to be able to launch that drug early. We know that in most cases, once the patients are on a clinical trial, you don't take them back off after their drug has been approved. Now companies are looking at two to four years during which they are going to pay for the patient. I don't think so. And they are not going to want to come to a country where the prices are set in such a way that they are going to be at a disadvantage. That's not to say we don't negotiate new prices. We can't negotiate in a way that's different from other countries.

We do want the federal government to reconsider what's happening in terms of those PMPRBs. We support wholeheartedly lower drug prices. We support wholeheartedly the necessity of making sure that Canada does not pay more than other countries. However, we cannot do it in such a way that it's different from other countries. That's going to actively discourage companies from coming in.

Sorry, I'm making this up as I go along now.

● (0955)

The Chair: You're doing great.

Dr. Durhane Wong-Rieger: I do want to speak to the special access program that was brought up. I think this is a huge problem for us here. Quite frankly, as Dr. Stewart said, we had 13,000 requests and only 48 rejections. Really? Should we have a system in which we have 13,000 requests through SAP? Is this the way to run access? I don't think so.

We need to consider how we can bring many more of those drugs into the approval process. I think the pathway that Health Canada has put forth, the modernization of the regulations, could in fact encourage that, but I can tell you—and this is not the time to tell you—in great detail why the companies hesitate and why, even after they now apply, there are such barriers in terms of the approval and reimbursement process that a company looks up and says, "I don't think so." That's a problem. I think this is a challenge for us.

Definitely, as my final comment, we would like to propose a national pharmacare program in which the federal government actually can take a huge role. It was promised to us initially in 2006 when the Liberal government was in place. We actually had a proposal put in front of us from the federal-provincial-territorial committee around a program for expenses for rare diseases. In 2012, 2014 and 2015 we've had continual promises that this would be happening. We've yet to see anything materialize, even though we advocate on their doorstep at almost every health minister's meeting.

This is something we would like to see as part of this national pharmacare that's coming forth. We've had great discussions with Dr. Hoskins, and they have encouraged it. We need to have a program that is going to be publicly owned. It could have a private component to it if they choose to, but patients should have the opportunity to sign up for a public program funded by federal and provincial governments that's going to be one program, one set of guidelines, one approval process, one set of databases in which we can enrol patients and provide drugs in a managed fashion that would allow patients to get access to drugs as they are brought in.

If we have a parallel process with HTA and Health Canada, great. At the time of NOC we should be able to open up this program. The federal government, we believe, needs to take a huge role in doing that and we can bring the SAP into it. There's no reason that SAP drugs should not be adjudicated as part and parcel of that. You have a clinical panel. You have experts. You have databases. We can actually manage an entire rare disease program, and it will put us at number one. It will be something. Nobody has an ideal program. We think Canada is in a good position to actually step up to that spot.

● (1000)

The Chair: I don't want to miss a word you have to say, but I have to cut you off. We're a little over time.

Dr. Durhane Wong-Rieger: I have finished. That's great.

Thank you very much, again.

The Chair: We do appreciate your comments, and we look forward to more when the questioning starts.

Now we go to Tammy Moore from the ALS Society.

Ms. Tammy Moore (Chief Executive Officer, Amyotrophic Lateral Sclerosis Society of Canada): Mr. Chairman and honourable members of the Standing Committee on Health, thank you for the invitation to appear before you today.

We appreciate this opportunity to address barriers to access to therapy. It is a reality our community finally has the opportunity to deal with, but it is a growing risk we see for the future. We were pleased to hear about some of the advances Health Canada is making, but I'm also going to share with you today an illustration of some of the issues that even in our limited experience we have seen give us further concern for the future.

I am here today representing Canadians affected by ALS. I wanted to invite Carol Skinner, a young woman and strong advocate from the ALS community, to join us here today. In fact, many of you have met her in the past, but she has to send her regrets because to be part of today's proceedings exactly highlights some of the issues associated with rare diseases like ALS.

Carol has a terminal illness. It's robbing her of her mobility, her ability, and her independence. To be here today, Carol would have needed her personal support worker to come in extra early to attend to the daily routines that most of us take for granted, such as, brushing our teeth, washing our face, getting our clothes on, tasks that would further be challenged by the fact that this meeting is happening early in the day and her muscles do not respond as readily as they might later in the day.

Carol's husband, Travis, would have needed to take the day off work to physically support her in attending resulting in lost wages in a family already burdened by the heavy cost of a costly disease. Carol's reality underscores that ALS is not just a terminal neurodegenerative diagnosis for 3,000 Canadians who are currently living with the disease, but it's a disease that impacts many more people. The emotional, physical and financial costs are devastating.

Each year, 1,000 more Canadians will be diagnosed and they and their loved ones will find themselves on a journey with the disease that in the course of two to five years will take away their ability to eat, speak, move and eventually to breathe as their motor neurons die.

Only 5% to 10% of people who are diagnosed have a hereditary link, and in those families, the disease devastates in each and every generation. We each in this room have a one in 400 chance in the course of our lives of having an ALS diagnosis.

The current situation in which there is almost nothing in the way of therapeutic options leads to an urgent desperation as people consider any options that will provide them hope, including those that put them at greater risk and financially drain their resources and challenge a health care system that has to pick up if something goes wrong.

With this context, for the remainder of my remarks, I ask that you consider what it would be like to be paralyzed by ALS. Please try sitting still and not moving a muscle until I'm done speaking.

We have several recommendations that we would like you to consider in access to therapies.

One is to create an environment that makes Canada a country of choice for new therapies throughout the spectrum from research and development, through clinical trials, through new drug submissions, through approval process and reimbursement. Another is to coordinate, streamline and increase transparency associated with those processes and timelines that enable patients to access drugs both before and after market access is granted. We also recommend that you please work with the provinces and territories to address the inconsistencies that currently result in the inequitable access across our country.

Our recommendations are grounded in the following principles: equity, timeliness of access, affordability and patient partnership. Every Canadian should have equitable and consistent access to high-quality treatments that are appropriate to their individual needs.

In regard to timeliness, Canadians should have access to the treatments they need in a timely manner. My population doesn't have time to wait. In terms of affordability, they should be able to afford both the treatment and the means to administer the treatment. Patient partnerships must be meaningful. They must be thoughtful and they must make a difference. Above all, we have to have transparency in the process.

As a member of the Canadian Organization for Rare Disorders, we broadly support the call for extensive stakeholder engagement and a strategy for the management of rare diseases. These recommendations and guiding principles are a result of the experiences we've recently had in our community and our engagement with organizations like CORD and the Health Charities Coalition of Canada, HCCC.

● (1005)

I would like to share with you some specific barriers recently experienced by Canadians living with ALS.

Some of you have met Norm. His situation demonstrates the lack of a streamlined process in clinical trials and pre-market access, which results in physical, emotional and system costs. Norm participated in a clinical trial in which there were no adverse events. Once the clinical trial concluded, the company was willing to provide the drug to him, but an application had to be made to Health Canada for an open-label extension. The process of approval took six weeks.

While this may not sound like a long time for any one of us, this could mean the difference in terms of the ability to speak or to move your hands. Unfortunately for Norm, during this gap in treatment his disease progressed with a loss of function. It directly resulted in two significant falls. The resulting injuries required hospitalization, including epidurals, to deal with the pain from the back injury.

Finally, after having to act as an active liaison between the two different decision-makers that were not directly communicating, Norm was granted an open-label extension. When safety of the therapy is not a concern, this type of delay and regulatory inefficiency is not acceptable. It creates uncertainty and unfairly and unnecessarily impedes the access to therapies.

In comparison, in the U.S., when the FDA approves a clinical trial, as long as there is no safety signal, that open-label extension is immediately available through the conclusion of the clinical trial protocol. This process avoids a potential gap in treatment, like the one Norm experienced that caused injury and loss of function. In Norm's case, that function cannot be regained.

Right now we are also seeing lengthy timelines and a lack of transparency in Canada's regulatory and reimbursement process, both of which affect patient access. In May 2017 the FDA approved Radicava as a treatment for ALS in the United States. After considerable lobbying of the company by patients, by ALS Canada, and yes, even by Health Canada, eventually, in March of 2018—it took almost an entire year—the company decided to put their drug through the regulatory process in Canada. Currently that drug is under priority review. Given the 180-day timeline, we expect that the decision is imminent.

In those 17 months since the FDA's approval, those in our community who could afford to do so have utilized Health Canada's personal importation process. They have paid out-of-pocket to import the drug. This method of access is not in the spirit of equitable access within a universal health care system, and it has put people at risk.

We are also very aware that even if this drug is given a notice of compliance, or an NOC, with conditions, it will not mean that the treatment is readily available, as we heard earlier today. We expect that CADTH may provide reimbursement recommendations to the provinces and territories by the year's end and that it will be sometime after that before actual decisions are made. But with no defined time frame and no transparency in the process, companies may find the lack of clarity not worth the business risk to consider a Canadian marketplace, which means that Canadians will not have access to new therapies.

In the 180 days during Health Canada's priority review period, 500 Canadians have died of ALS. How many will die awaiting the CADTH decision? After that, how many will have to die while they're awaiting the availability through a publicly funded drug program? We are dealing with a community that measures time by loss of their own function and by the number of members who will die during this process.

Access issues do not end even once the drug is in hand. It also means creating a system where patients can have the drug administered equitably, regardless of where they live or their financial means. Even though many in our population were able to access this drug through their own initiative, many face challenges with getting it infused. This drug requires administration through an IV. It's typically 10 days out of 14, followed by 14 days without the drug, and then the cycle repeats. Provinces had different policies and approaches to managing the infusion, and many people had to pay additional costs to have the drug infused by private clinics or nurses. In some provinces, the situation was so dire that the health care system wouldn't support the drug administration. While people had the drug sitting on their kitchen table, they were on Kijiji looking to see if they could find someone who would be willing to infuse the drug.

● (1010)

This puts an already vulnerable and desperate population in a risky situation that could end up having an even higher cost to the health care system due to adverse events.

Our concern as we look to the future is that even with the pCPA process, which is designed to establish a consistent funding approach across the provinces, we will continue to see differences not only in reimbursement decisions, but in standards of practice.

Also, of course, a new effective treatment cannot improve the health outcomes of Canadians if the drug is delayed in coming to Canada or, alternatively, does not launch in the Canadian marketplace at all. Canada, with its relatively small population, must be a competitive player in attracting manufacturers to bring their therapies here throughout all stages of the therapeutic pipeline.

There are more ALS therapies on the horizon. We do not wish to see the challenges of the last 18 months repeated as other therapies come forward. We cannot leave a desperate and vulnerable population without hope when they can see it just across the border but don't have the physical or financial means to access a therapy that could save their lives.

One thousand Canadians are dying of ALS each year. How many more Canadians will die before our health care system responds to the needs of Canadians who are unfortunate enough to receive an ALS diagnosis?

The Chair: Thank you very much.

We'll go to questions now. We'll start with Dr. Eyolfson for seven minutes.

Mr. Doug Eyolfson (Charleswood—St. James—Assiniboia—Headingley, Lib.): Thank you, Mr. Chair, and thanks to all of you for coming.

Ms. Wong-Rieger, thank you for coming. It's good to see you again.

You've made some mention of our pricing policies and how there have been concerns that they are making this a non-competitive environment. As you may be aware, Canada pays the second- or third-highest costs for drugs in the world. Many other countries are paying less and have universal pharmacare systems. Some are a mixture of public and private. Others are completely public. New Zealand, for instance, is a country with a small population over a reasonably large area. They have a universal program. They pay much, much less for drugs. The government buys commonly used drugs for probably less than a tenth of what we pay.

Are countries like this that are paying much less for drugs having problems with the marketplace not wanting to develop new drugs? Are people in New Zealand having trouble accessing them? Are people throughout the EU, which is paying much less for drugs, having trouble accessing drugs for rare disorders?

Dr. Durhane Wong-Rieger: The challenge in terms of accessing drugs for rare disorders are many, as you say, and they vary in different countries. As we say, nobody has an ideal system.

When we look at New Zealand, let's be real clear. Nobody with a rare disease gets access. We know the New Zealand Organisation for Rare Disorders very well, and almost nobody gets access to any treatments. For his two children, the past president never got access to a drug that actually was quite readily available both in Australia and in Canada. If you want to use New Zealand as an example, it's absolutely not going to be supported in terms of rare diseases. Their formulary is very limited. New Zealand is just off the table in terms of any kind of comparison.

You can look at other countries. Obviously, there are some countries that do it better. Partly what we will recommend is looking at the countries that do it well.

How do we bring these in early on? How do we negotiate a price? Part of the challenge in terms of pricing in Canada, as you know, is that we do have a private market system that actually does not negotiate as one in the same way that the public system could. The difference in what we pay privately and what we pay publicly is huge.

Quite frankly, the reason we get such great prices publicly—and we know when we look at those prices that they are much lower than many other countries would get for those drugs—is that they can offset it with the private. If we're going to have a harmonized system, one system, then we're going to have to look at somewhat of a blended price. We agree.

We do agree that the prices can come down lower in Canada, and they should come down lower. We do not agree with what the regulatory reforms are suggesting, that is, that we put a value-added proposition up front to look at the list price. Again, this is not what other countries do. It will put us at a disadvantage.

We agree. Negotiate better in order to get a price that's actually going to bring us down, no doubt about it, but one of the ways to do that is to negotiate as a country, to negotiate as one drug plan. We believe that would be helpful. Also, definitely, bringing in the drugs earlier on and then negotiating those prices, the way Germany does, the way France does, the way the U.K. has been able to do, and where we've seen Australia going...these are some of the models. We think Canada could do quite well if it follows what it's doing but recognizes that the differential in pricing has to do with the fact that we do have a large private component.

That's a different question in terms of how we want to address this, and it's something that Dr. Hoskins is trying to address for pharmacare, but quite frankly that is not actually the situation with regard to public prices in terms of rare diseases. We negotiate pretty hard on those and we get them down pretty low. The problem is that it takes two to four years to negotiate them. There's where the tragedy lies. I think we can have a better process that can do it in a much shorter period of time if in fact we look at the negotiations at the time of the NOC, with the HTA having been done appropriately at that point.

● (1015)

Mr. Doug Eyolfson: If you were to pick a nation that would be the ideal for the best approval for rare diseases, which nation would you pick?

Dr. Durhane Wong-Rieger: I think we could do a great job in Canada by choosing the best from all of the countries. We can look at what—

Mr. Doug Eyolfson: Let's nail it down to one.

Dr. Durhane Wong-Rieger: I don't think we can nail it down to one, because nobody does it perfectly.

Canada, as you know, is a wonderful hybrid. We can learn from everybody. I think that's where we want to go.

We can look at what France does in terms of providing it immediately. We can look at what Germany does: have a managed access scheme that brings in the drugs, and we'll renegotiate the price after a year. We can look at the U.K., which says it has a highly specialized approach for ultra-orphan drugs.

We can adopt all of those in our proposal for a managed access scheme in Canada. Bringing in those drugs and making them available to people under a monitoring system, with information that will allow us to decide who can continue and what the right pricing is over a period of time, we think picks up all the best of those.

Canada can step up, and I think other countries can say, "Why don't we do what what Canada does?"

Mr. Doug Eyolfson: Thank you.

We've talked about pharmacare, and I know you gave us some testimony on that. I won't bother there.

There's a recommendation regarding the government supplying CADTH with additional funding to undertake its capacity to review high-cost specialty drugs and develop expertise to support these negotiations.

What other recommendations would you ask this committee to make to improve access for rare diseases?

Dr. Durhane Wong-Rieger: First of all, we want to make sure we do not have the barriers that the proposed reforms to the PMPRB would have, and that is to discourage countries from coming in.

I think we want to talk about having a national program that would allow us to bring in all of those patients. The numbers are small, so we're not going to be able to....

Most of these drugs have to come into specialty clinics, so we have a proposal for a rare disease strategy that would also include centres of excellence. They would include the ability to do what we call a life-cycle approach. We can bring in the drugs. We can identify early on, as we're bringing them in, which patients are going to be immediately eligible—like those with ALS—and which ones are not at all going to be eligible, set up a monitoring program for them to have access to it, and then over time, as we are learning from those patients who are on it....

In the old days, we would call it post-market monitoring. As Cathy talked about, under the new bill, Bill C-17, we can enforce those kinds of post-market monitoring programs that we can learn from.

We have a lot of tools at our disposal and, quite frankly, we do not necessarily need to invent them. We can look at some of the model programs we've put together. Canada knows how to do this. We already have these kinds of monitoring programs with very specific drugs, but we want to be able to make that the standard, make it so we can do it nationally, which we don't necessarily do well, and then not wait until we've gone through two more years of negotiations before we implement it.

We know how to do it. We just need to make sure we put that template in place and have the specialty clinics that are there also able to support the use of them, and, as she talked about, the administration of them, and the ongoing data collection to make sure they're used appropriately and, at the end of the day, price readjustment.

● (1020)

Mr. Doug Eyolfson: Thank you.

The Chair: Thanks very much.

Mr. Lobb.

Mr. Ben Lobb: Thank you very much, Mr. Chair.

We can maybe see where this goes, but I think it would be great if we could have the officials back to conclude this round of meetings and studies to see if they have had any thoughts after we've gone through this and whether they'd like to add to this discussion.

Durhane, you made a great point in the beginning about Dr. Stewart. I know they're here to do a great job and everything else, but I can tell you, the number of people who call my office who are not happy with the way Health Canada.... It's not to blame a government; it's just to say the state of the situation. They're not happy. Then I have drug companies that come to visit and have meetings with me. They're not happy. Yet, Health Canada sits here and tells us everything is going pretty well. It seems to me there has to be a disconnect here.

There are 13,000 SAP applications, and I think they made a comment today that they're knocking off about three per year or something like that. They're getting three of these companies to come in and have licensed drugs.

How do we convince these companies to go from being in the SAP to being licensed? What do we have to do as Canadian parliamentarians, Health Canada or the Government of Canada?

Dr. Durhane Wong-Rieger: Certainly we believe that the modernization of the regulatory frameworks has provided some tools that can make that happen.

You need to look at where the different barriers are. In some cases companies don't come, because these drugs have been around for a long time, and there's no new evidence in terms of how well they work. If you have a process that says, "We need to have you submit evidence based on clinical trials on how these work", these companies are saying, "I'm not going do a clinical trial for Canada; we have 20 years of evidence, in terms of real-world usage." That hasn't been enough sometimes to get them to get an NOC.

That's number one. We need to be able to accept real-world evidence the same as the FDA does, to say, "Okay, that can happen".

The second thing we need to look at is that in some cases these companies aren't coming in because it costs a lot. This is a generic drug by this time. They're making peanuts on it. In fact, sometimes they're making less than peanuts on it, because it's lower than their costs. Now they would have to pay an application fee of how much in order to get this drug approved. After it gets approved, we have companies that are caught in a loop that they must now submit to CADTH in order to get it reimbursed. Well, hello, it's already being reimbursed as an SAP drug. Now we want them to pay 70-some thousand dollars to get us to reapprove it. We're not going to negotiate a price, so why would they do this?

We put in these bureaucratic obstacles that do not need to be in there. I just had a company that called me again and said, "Can you please just get Health Canada to accept the IND that we have in the States, develo,.ed for a clinical trial? Do not make us write a separate protocol for you. I have five babies that are waiting for treatment. Can we not do this right away?" I'm kind of thinking that it should be able to do it, but we haven't been able to figure out how to remove those kinds of barriers. It can be done, but I think we need to have an even more enlightened approach.

I don't fault the individuals who are there. I really have huge respect for everybody who was here at this table. They work with us as hard as they can. Good God, we keep throwing land mines in their path and tell them to keep jumping.

I think this is the challenge, and at the reimbursement end as well. This is a problem for us. We have companies.... Actually, let's talk about the drug Cystagon. The company brings it in. It's been here for years. They have a certain price, and now we're saying to them, "We'd like you to have an NOC on it so that we can get it out of the SAP." They're saying that in order to do that, they are going to have to raise the price of the drug. Then, we have one province—I won't tell you which one; I should tell you which one it is—that says, "Oh, you've raised the price of that drug. We don't want to reimburse it any longer, because it used to be cheaper." Now we have to intervene with the province, to say this is ridiculous.

● (1025)

Mr. Ben Lobb: I would call it a Ponzi scheme. I think that's what you're going to see, a rare disease Ponzi scheme amongst the pharmaceuticals.

A drug company visited me about this. It bought a company that had one product and paid $800 million for it. They said to me that they would now have to charge a certain amount to make their money back. Who told them to go and spend $800 million on this product?

That's in the same breath with Mylan. That's what they've done. They sold the rights or whatever. Whoever it is who bought them, Recordati or whoever it is, can now justify saying that it is going to cost more money, that it is going to be extra.

I hear what you're saying, and I can imagine all my colleagues here are so frustrated that it's right there; I don't understand how it can't get done. I'm sure we're all very frustrated with that.

I'd like to ask Ms. Moore a question in regard to the clinical trials.

Any member of Parliament who's been doing this long enough, whether it's in our social circles or just out and around, we've all met people who have had ALS. It's terminal, and it's pretty tough to see that take place. When it's in a situation like that, what do we have to do to get it from six weeks to virtually immediate?

Ms. Tammy Moore: It would be a simple alignment, the same as what they have with the FDA in the U.S. When the clinical trial protocol is approved, it automatically gets open label extension, unless there has been an adverse event or something to signal safety issues associated with it. There should be no reason that a separate application has to go through for an open label extension once the clinical trial protocol has been met. It seems like a simple solution. I'm not certain.

Mr. Ben Lobb: I have another question for Durhane.

One comment that Dr. Stewart made—it was kind of interesting the way the two witnesses were talking—was that we'll respect the commentary from the physician and the patient, because in some cases they know best. At the same time, Dr. Stewart was saying, and I'll paraphrase, that may be, but you're still going to have to have your physician, etc., prove without a shadow of a doubt that they have to stay on this drug.

You see thousands of people throughout your career. Is that the right approach to take?

Dr. Durhane Wong-Rieger: Again, I think the way in which the process works penalizes the clinician and the patient, and we don't have to do it that way. I think that if you have patients, as you've rightly said, who are on a lifetime drug, the necessity to reapply every three months because it has not actually been given NOC makes no sense whatsoever. It's the anomaly in which the SAP is written. We get it, in terms of the way the law is written, but that can actually be changed.

Our recommendation is to bring those drugs into a national rare disease pharmacare program. We can deal with them in the same way as we deal with all of the drugs that have uncertainties. You have a panel. You set up the guidelines in the protocol, and you make sure that the patient and the clinician are fitting within that protocol. The monitoring sits within that program. It doesn't have to be bumped up. As long as you say there's no adverse event, as long as the patient is responding appropriately to it, and as long as the physician can attest to it, then the patient just stays on the therapy.

There should not be the necessity of having to go back every three months to reapply. As all the clinicians say to us, they don't get paid to do this. I'm not saying that in any kind of derogatory way. They have hundreds of patients. This is actually taking away from them being able to do something else.

So yes, we could do that, and I think what we want is to go back and ask what the real experience is here. In some respects, I think what we often hear is a request for a modicum of common sense when we're doing these things. If we could just introduce that, I think that would help a whole lot in terms of what we're able to do.

Mr. Ben Lobb: I know my time is up, but I would just like to say that many issues on Parliament Hill are political, whether it's your views on taxes or something else, but I don't think this one is. Whether you're NDP, Conservative, Liberal, or other, you should want this to be best for everyone who lives in your area.

● (1030)

Dr. Durhane Wong-Rieger: Thank you.

Mr. Ben Lobb: Let's hope we have some good recommendations that come out of this.

The Chair: Thank you, Mr. Lobb.

Mr. Davies.

Mr. Don Davies: Ms. Moore, ALS Canada's website says, "There are no significant sources of Canadian ALS research funding other than ALS Canada, resulting in a need for directed support from other sources like the federal government."

How much funding does the federal government currently direct towards ALS research?

Ms. Tammy Moore: There is nothing specifically directed towards ALS research in Canada. We were fortunate in the past to be able to secure matched dollars through the Canadian Brain Research Fund, in partnership Brain Canada, but those were matched dollars, and the only reason we had those dollars available was because of the ice bucket challenge. Other than that, we are a Cinderella disease, and we can't get out of that cycle.

We provide for gaps in the health care system. Our societies across the country work in a very strong collaborative model, and we are filling a gap within the provincial health care systems to provide hospital beds, wheelchairs, ceiling lifts, ramps, and things that enable people to stay in their homes, where they are best cared for, and out of the health care system.

We're grassroots fundraising organization, and we're now back to the fundraising levels we were at prior to that one anomaly, so we are back to about $2 million that we have to direct towards research. There is not the same opportunity going forward for the $10 million that we secured from Brain Canada, because we don't have a massive pool to draw from.

Aside from that, our researchers have the opportunity to apply to programs like CIHR. However, because we are a relatively small population, both in terms of the number of Canadians living with ALS and the number of researchers, we have a very small opportunity for success within CIHR. Once again, it becomes this Cinderella disease. How can we possibly break this cycle using population-based research funding models or population-based research support?

I would even challenge that as we're talking about the support of clinical trials, we're talking about registries. The Canadian Institute for Health Information does not get down to the level of ALS when looking at a neurodegenerative disease, so the data collection on ALS within Canada is done by societies like mine, in partnership with donor-funded, volunteer-based organizations across the country. In P.E.I. there are three volunteers supporting the people living with ALS in their province. They are trying to help collect data to support advocacy efforts and to support clinical trial information. We need other systems in place to support rare disease, and diseases like ALS.

Mr. Don Davies: Thank you.

Ms. Moore, you mentioned some of the difficulties with patients facing significant out-of-pocket costs.

Ms. Smith, have you faced any significant out-of-pocket costs? I'm curious about what the financial impact has been on you. Also, what recommendation would you give this committee if you were health minister or prime minister?

Ms. Maureen Smith: Oh boy.

Mr. Don Davies: What would you as a patient suggest to us would make a big difference—

Ms. Maureen Smith: As a good Canadian, I file my taxes every year and I file my medical expenses. My out-of-pocket medical expenses vary between $7,000 and $10,000 a year. I'm 40, so I've been paying those costs for at least the last 20 years, because, first of all, I travel to Toronto for care. My disease is ultra-rare, so I see a specialist there who specializes in that area. I spend a substantial amount of my own money. I'm very fortunate to have private insurance with a catastrophic clause in it, so that helps a lot. But even with that, I'm still $7,000 to $10,000 out of pocket.

I have a lot of ideas about recommendations, but I think the thing that strikes me the most is that there's no equity across the country. It's very difficult for a patient to know that someone in B.C. or someone in another province has exactly the same condition as you and receives treatment when, maybe in your province, you don't. People who don't have rare diseases or who don't deal with drugs are flabbergasted by that. We're all Canadians, and they seem to think that universal coverage in hospitals extends to drugs. There's very little understanding of that until you're in that situation yourself.

What's happened with the Internet is that everyone is connected now. Twenty years ago, you didn't know what was happening anywhere else. You were in your own little bubble and you just kind of accepted it. Now, today, you know what's happening worldwide, so to have a catastrophic rare disease and to know that there's treatment out there and that even some of your fellow Canadians are getting it and you're not is extremely difficult to accept. To me, it's not a Canadian value. It's just not how we work as a society.

I think that whatever you can do to try to bring some equity into the system and to make sure that all Canadians are served would be top of mind.

● (1035)

Mr. Don Davies: You mean like a call for federal national coordination or leadership, which leads into my next question, which is for Ms. Wong-Rieger.

You said three million Canadians have rare diseases, not including their families. I wrote down that rare diseases are quite common, it seems to me. That being said, you mentioned that basically we've heard continuous promises from the federal government since 2012. You heard the excerpts I read about how the federal government, Health Canada, announced it was developing an orphan drug framework in 2012. Consultations were to be complete by 2017, but in October 2017 under the current government, all references to that framework were deleted, and now it's been wrapped into something else.

Why is it so difficult to get action from the federal government, and what's your comment on Health Canada's apparent scrubbing of the system that began in 2012 and a complete retooling, rebeginning of this process in 2017 with now a new target date which, I think we heard from Health Canada, is 2021? We are at about nine or ten decades before getting federal leadership on this. What's your comment on that?

Dr. Durhane Wong-Rieger: I'm trying to decide whether I should really speak honestly about what I think or whether I should just give you the politically correct response.

Mr. Don Davies: Speak honestly.

Dr. Durhane Wong-Rieger: Speaking honestly, as we were dealing with this regulatory framework, we kept getting promises that it was going to be submitted, and we kept hearing that the provinces are concerned about it because they're afraid that by having this framework, we're going to increase the number of rare disease drugs that they're going to have to pay for, which was a tragedy.

Part of what we also said was that we want to be able to support having research and development in this country. That's what this framework would have done. We do have drugs for rare diseases that are discovered in Canada and then by the time they get into clinical trials, they've been picked up and gone elsewhere because we do not provide the incentives to nurture that development, as you heard from Cathy and as you'll hear from Tammy.

At the end of the day, we're just net payers. We do not have a research infrastructure, but we also don't have a development infrastructure that says.... When Europe introduced their orphan drug act in 1999-2000, some 15 years after the U.S., they didn't do it just to serve their citizens. They did it because they said all the research and development monies for orphan drugs were going into the U.S. They needed them in Europe, and they developed that framework to make that happen. That was part of our hope. Can we support research and development in Canada as well so we're not just net buyers, so we're net contributors, and if we're developing some of those drugs, some of those profits come back to Canada? We have not put ourselves in that position, so this is a real challenge for us.

Yes, there are lots of challenges. Part of that framework would have supported that. It didn't even go far enough to do that. We thought it was a good starting point, so let's built on it. It was also a signal to the world that Canada is open for business in rare diseases and orphan drugs. Come and develop with us here. Let us support that.

We hear this innovation mandate from this government and we laugh and say that on the one hand, we're talking about it and on the other hand, we slap it away, including what's happened with the regulatory reforms on pricing. On the one hand, we say we want to make Canada first, that we want to encourage the innovation to come here, but on the other hand, we say let's put up big barriers so nobody wants to come here first. Really? You wanted me to speak honestly.

My problem is I think there's schizophrenia here. Can we get it together? Are we going to be number one? Are we going to support innovation? Are we going to encourage research and development in this country? Are we going to encourage drugs coming in and having clinical trials here and making them available to people or are we not? On the one hand, we say we are and we are doing some things to make it happen, and on the other hand, we keep creating more and more barriers and making it more and more challenging for us. At the end of the day, the poor patients are the ones who lose here.

● (1040)

The Chair: We'll move to Ms. Sidhu now.

Ms. Sonia Sidhu: Thank you, Mr. Chair.

Thank you, witnesses, for being here, and thank you for your advocacy. I have one question.

I know families who suffer from ALS. Are they going through any social isolation? We heard about barriers. We heard about challenges. Are there any support systems out there? Anyone can answer.

Ms. Tammy Moore: Yes, you can imagine that as a loved one becomes gradually paralyzed, there are the very physical mobility aspects. That is where an organization like ours—and in your riding it would be our organization—would help to fill a gap left by the health care system.

These people are in their homes and as they're losing their independence, they require supports: hospital beds, wheelchairs, ceiling lifts, things to be able to get people in and out of their homes. If those aren't in place, then they're even further isolated, but again, as their care needs increase, our health care system isn't keeping up with that, so often a caregiver, someone within the family, will have to stay home.

Mr. Davies had asked about the costs associated with it. We had done a study and we know that the costs associated with ALS are between $150,000 and $200,000 in the course of the two to five years that someone will live with this disease. We're talking about loss of income as well. The social isolation as well as the financial constraints become even more significant, so our population is making very hard decisions: "Do I bother modifying my home for the six months I'm going to enjoy it? I'm going to have to take the equity out of my home, out of my child's education fund to be able to support this," or unfortunately, "Will I have to make other choices about how I'm going to live out my final days as a result of those hard financial considerations?"

Social isolation is an important aspect that we also help to support. Again, we are a donor-funded organization that has people in our communities around the province. We work hand-in-hand with the ALS clinics, but once somebody is diagnosed, they're immediately signed up with our societies and our people will go to their homes and help them to start navigating their journey. We're providing psychological support groups so they're able to be with people who understand what they are going through. We have many supports in place like that to be able to, but we're a donor-funded organization. Should a charity have to fulfill this role in our society?

The Chair: Ms. Sidhu, we have to call it quits now because there's another group waiting for the room. Thanks very much. I'm sorry to cut you off.

Ms. Sonia Sidhu: Thank you.

Thanks very much to our witnesses. You're very passionate and very informative, and you've helped us a lot.

The Chair: Thank you.

With that, we adjourn meeting number 112.

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